



## HTAi Patient and Citizen Involvement in HTA Interest Group (PCIG) E-Bulletin, April 2016

*Enhanced quality and relevance of HTA through patient and citizen involvement*

Welcome to this month's E-Bulletin

We wish those of you going to Tokyo for the HTAi Annual Meeting safe travel!



**Unlocking the Value Potential of New Technologies in Health Care** Are Evidence-based Decisions Possible?

**Waste in Science** How Does this Affect the Perceived Value of a Health Intervention? New Ways of Measuring Value

- **Global Experiences in Universal Health Coverage** Potential Barriers and Enablers for the New Era of Valued HTA in Asia

### Informing Health Care Decisions with Values and Evidence

The use of value-based approaches to the issues in health care, especially to reimbursement or pricing has been well established across western countries and over the last decade Asian countries have been seeking their own solutions. Asian health care systems are very diverse, representing cultures, political systems and economies from more than 30 countries with varying histories. Despite the diversity in the region, there has been enormous growth in health economics and outcomes research driven by the desire to establish universal healthcare insurance coverage, the need for rational allocation of scarce resources, and a desire for government to provide leadership in HTA. This has been supported by the availability of HTA professionals and faculties through international networks and underpins the partnership between HTAi and the WHO. All countries experience their own health system challenges, from implementing new technologies, addressing inequity to being pre-emptive in meeting the challenge of a rapid shift in demographics like age. These have the potential to threaten the sustainability of current health insurance systems, requiring greater deliberation on the issues of a value-based approach to policy decision-making and implementation. There is a need for informed discussion by a wide range of stakeholders on the use of advanced and expensive medical technologies such as personalized medicine, molecular targeted drug therapy, cancer treatment, regenerative medicine, heavy particle radiotherapy (and the facility) to name a few.

The Annual Meeting in Tokyo will provide an opportunity to consider:

- The role of HTA in universal health coverage
- The rationale for subjective / political adjustment in decisions
- The dilemma of cost-saving versus investing for innovation
- Scientific evidence versus pragmatic decision making
- Dealing with a lack of epidemiological data
- The utilization of information technology in HTA
- The role of patient participation in decision making
- Cultural differences (e.g., utility measurement, expectations to the government, societal consensus).
- Country-specific HTA
- International data sharing and evidence transferability

### Starting as it means to continue:

The HTAi Patient Panel has set up a Google Doc Register of where it has had an impact and added value. One way has been to complete a questionnaire on how their patient organisations contribute to HTA, to inform

our Interest Group workshop in Tokyo. Our pCODR/pERC Patient representatives from Canada also completed the questionnaire, making an interesting comparison. Some of the Panel members will be in Tokyo contributing actively to our programme, where we will be working together to enhance the quality and relevance of patient involvement in HTA internationally.

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### **CADTH Annual Symposium**

Carole McMahon and Jo Nanson were privileged to be two of the patients whom the Canadian Agency for Drugs and Technologies (CADTH) sponsored to attend the CADTH conference in Ottawa. It was great, says Jo.

While we were there, the patient representatives from the *pan-Canadian Oncology Drug Review* (pCODR), the Common Drug Review (CADTH's committee for all drugs except oncology drugs), the Ontario Drug Review program, CADTH's Technology Committee and the British Columbia Drug Committee were able to meet and share common experiences and goals.

Jo and Carole are patient representatives on the pCODR Expert Review Committee (pERC)

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### **INTEGRATE-HTA**

The European (FP7) INTEGRATE-HTA project developed concepts and methods that enable a patient-centred, comprehensive, and integrated assessment of complex health technologies which were applied in a palliative care case study. Both lay and professional stakeholders were involved throughout the HTA process.

The INTEGRATE-HTA project team will present the results of the project at the HTAi 2016 Annual Meeting in Tokyo (10-14 May). The presentation "An Integrated Perspective on the Value Of Health Technology. Results of the Integrate-HTA Project" will be presented in the Oral Session 07 Methods & Decision Making in HTA on May 12th (14.30-16.00).

In addition, the following publications are recently published or in press:

Lysdahl, Kristin Bakke, Wija Oortwijn, Gert Jan van der Wilt, Pietro Refolo, Dario Sacchini, Kati Mozygemba, Ansgar Gerhardus, Louise Brereton, and Bjørn Hofmann. Ethical analysis in HTA of complex health interventions. *BMC Medical Ethics* 17, no. 1 (2016): 1.

Lysdahl, K.B. Hofmann, B. Complex health care interventions. Characteristics relevant for ethical analysis in health technology assessment. *GMS Health Technology Assessment* 2016, Vol 12, ISSN 1861-8863.

Rohwer, A., Pfadenhauer, L., Burns, J., Brereton, L., Gerhardus, A., Booth, A., Oortwijn, W. & Rehfuess, E. (in press) Logic models help make sense of complexity in systematic reviews and health technology assessments. Accepted by *Journal of Clinical Epidemiology*.

Refolo, P., Sacchini, D., Brereton, L., Gerhardus, A., Hofmann, B., Lysdahl, K.B., Oortwijn, W., Tummers, M., van der Wilt, G.J., Wahlster, P., Spagnolo A.G., "Why is it so difficult to integrate ethics in Health Technology Assessment (HTA). The epistemological viewpoint." Accepted by *European Review for Medical and Pharmacological Sciences*

Brereton, L., Ingleton, C., Gardiner, C., Goyder, E., Mozygemba, K., Lysdahl, K.B., Tummers, M., Sacchini, D., Leppert, W., Blaževičienė, A., van der Wilt, G-J., Refolo, P., Denicol, M., Chilcott, J., Dehlfing, A., Hentschel, K., Oortwijn W., (in press) Lay and professional stakeholder involvement in scoping palliative care issues: methods used in seven European countries. *Palliative Medicine*

Please keep up-to-date on our follow-up activities through our project website: [www.integrate-hta.eu](http://www.integrate-hta.eu).

Contributed by Louise Brereton & Wija Oortwijn, on behalf of the INTEGRATE-HTA project team

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### **An informal meeting in London UK**

Meanwhile a good number of the PCIG taskforce developing a Roadmap for HTA within the development cycle of a new technology took the opportunity to meet in London for the day on 25 April. Sophie Staniszewska organised the venue and each person found their own way there for a productive day.

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### **New SMC videos to assist with patient group submissions**

The Scottish Medicines Consortium (SMC) has produced two short videos to help patient groups with their SMC submission. Both videos can be found on SMC's website. The first one features an interview with Geraldine Mynors, a patient group representative from Attention Deficit Hyperactivity Disorder (ADHD) Parent Support, who recently provided a submission to SMC. Geraldine explains how her group went about preparing their SMC submission and provides advice on how to maximise the impact of patient group submissions to SMC:

[http://www.scottishmedicines.org.uk/Public\\_Involvement/Submission\\_form\\_and\\_guidance](http://www.scottishmedicines.org.uk/Public_Involvement/Submission_form_and_guidance)

The second video features David Standley, who is an SMC Public Partner (trained member of the public and SMC committee member) delivering the presentation on behalf of ADHD Parent Support, at an SMC Committee Meeting: [https://www.scottishmedicines.org.uk/Public\\_Involvement/Public\\_Partners](https://www.scottishmedicines.org.uk/Public_Involvement/Public_Partners)  
The videos can also be accessed on the Homepage: <https://www.scottishmedicines.org.uk/Home>

The ADHD Parent Support Patient Group Submission document to SMC can also be found on the SMC website as an example of good practice:

[https://www.scottishmedicines.org.uk/Public\\_Involvement/Submission\\_form\\_and\\_guidance](https://www.scottishmedicines.org.uk/Public_Involvement/Submission_form_and_guidance)

Jen Dickson, Scottish Medicines Consortium Public Involvement Co-ordinator

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### **The Genetic Alliance UK series of Charters on access to medicines**

*Scotland:* Genetic Alliance UK has published its charter on Patient perspectives and priorities on access to medicines for rare conditions in Scotland <http://www.geneticalliance.org.uk/scottishcharter.htm>

*NHS England:* It also has a Charter for NHS England (October 2014) for rare conditions

<http://www.geneticalliance.org.uk/nhsenglandcharter.htm>; and

*NICE, UK:* Patient perspectives and priorities on NICE's evaluation of highly specialised technologies Patient Charter at [www.geneticalliance.org.uk/hstcharter.htm](http://www.geneticalliance.org.uk/hstcharter.htm)

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### **Some items of interest form the European patient advocacy space**

A recent news piece from the European Medicines Agency (EMA) on "[Integrating patients' views in clinical studies of anticancer medicines](#)" acknowledges the importance of bringing the perspective of patients on their disease and the treatment they receive to the assessment of benefits and risks of cancer medicines.

A feature article from Politico: [Cancer groups win initial backing for binding HTA](#) highlights the European Cancer Patient Coalition's (ECPC) long and persistent policy work with our champion Members of the European Parliament (MEPs) to change the European legal framework related to the approval of cancer drugs.

If you want to read more about the amendments, and why the regulation 726/2004 is fundamental to grant fast access to innovative medicines, [click here](#).

Kathi has also been involved in the recent establishment of the Hellenic Cancer Federation, bringing under one roof 24 cancer patient organisations. She is President of that organisation, Vice President of the European Cancer Patient Coalition, and a member of our Steering Committee.

Kathi Apostolidis

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### **PCIG Members let you know about recent publications and activities**

Kristina Staley, TwoCan Associates, England shares with us:

It's not evidence, it's insight: bringing patients' perspectives into health technology appraisal at NICE by Kristina and Caroline Doherty. Research Involvement and Engagement 2016;2:4

Health technology appraisal involves reviewing the findings from clinical trials and economic data to produce guidance on how health technology should be used. This task is carried out by appraisal committees in NICE.

One of the several ways in which patients can feed their views into these committees is via a written patient statement. We asked nine committee members about what difference the information from patients makes to their decision-making. The Committee members reported that written patient statements offer a different perspective when reviewing the clinical and economic data. This can have a profound impact when a committee draws conclusions based solely on the data, which may not reflect the reality of patients' lives. The patients' and carers' input provides meaning to the data, 'bringing the numbers to life'. It identifies if the technology has any wider impacts than what's been reported in the clinical trial, and also if the trial has measured what's important to patients. We conclude that the written patient statement adds value to the decision-making process by helping Committee members to make sense of the clinical and economic data-it makes them look at the evidence 'in a different light'. Patients' stories are very effective in this context, because they have the power to communicate and to challenge Committee members' assumptions. Understanding this difference between analysing research evidence and drawing on patients' insights is important in thinking about what's needed in a written patient statement and the best way to obtain it.

<http://researchinvolvement.biomedcentral.com/articles/10.1186/s40900-016-0018-y>

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From Jackie Street, Australia:

Our paper on involving patients in health technology assessment in April's Health Expectations: Involving patients in health technology funding decision in Australia – stakeholder views. Lopes et al. 2016

<http://onlinelibrary.wiley.com/doi/10.1111/hex.12356/epdf>

Sally Wortley, also from Australia:

Has just had the following paper published in the Australian Health Review - Community views and perspectives on public engagement in health technology assessment decision making.

<http://www.publish.csiro.au/paper/AH15221.htm>

A perspective piece by Sally and three other HTAi members is on its way in this journal: Moving beyond the rhetoric of patient input in health technology assessment deliberations

[http://www.publish.csiro.au/view/journals/dsp\\_journals\\_pip\\_abstract\\_Scholar1.cfm?nid=270&pip=AH15216](http://www.publish.csiro.au/view/journals/dsp_journals_pip_abstract_Scholar1.cfm?nid=270&pip=AH15216)

Sally is attending the HTAsiaLink conference being held on the 3<sup>rd</sup> – 6<sup>th</sup> May. It is a closed meeting but for those going she is presenting on Thursday 5<sup>th</sup> May. The topic is: '*Tailoring public engagement to HTA decision-making: a discrete choice study*'.

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Holtorf, Anke-Peggy and Bertelsen, Neil. 2016. "Moving Center Stage: Patients Claim Their Role in Healthcare." *Farmeconomia. Health Economics and Therapeutic Pathways* 17 (1): 3–6.

<http://journals.edizioniseed.it/index.php/FE/issue/view/263>

And Deidre DeJean

has published on the limited qualitative research available in the literature used to inform HTA, only slightly more than 1% of all published research on chronic obstructive airway disease and early breast cancer topics.

Qualitative Health Research 2016 Apr 26

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Thomas Morel provides a link to the latest issue from Health Affairs that focuses on patient engagement topics and use of evidence:

<http://content.healthaffairs.org/content/35/4.toc>

One paper of particular interest is by Carman KL et al: Understanding an informed public's views on the role of evidence in making health care decisions. *Health Affairs* 2016; 35 (4): 566-74.

It is a report from the Community Forum Deliberative Methods Demonstration project, sponsored by the US Agency for Healthcare Research and Quality. The project team held 76 deliberative groups involving 907 people overall (August to November 2012).

Participants perceived evidence as essential to quality health care. They also believed personal choice or clinical judgement were important. Doctors were seen as having an important role in discussing evidence with patients and deciding whether individually to follow the evidence or not. Participants saw limitations in evidence as: evidence changes over time, is often based on studies excluding specific subpopulations (such as by age, ethnicity), and lacking clarity.

They therefore considered that evidence may not be as important as patients' personal preferences or convenience, doctors' clinical judgements, concerns about out-of-pocket spending, for example. Many participants were sceptical that purchasers, payers, providers would prioritise evidence of medical effectiveness because of their respective financial interests. Evidence of harm was more compelling than of effectiveness

The populations studied seemed to be well informed as shown by responses to the survey question on if they thought it was important/very important for people to ask their doctor about research results related to a medical problem, which was 90% 'yes' before deliberation and 93% after deliberation.

PCORI has made a number of these papers available on its website [http://www.pcori.org/events/2016/health-affairs-briefing-patients-and-consumers-use-evidence?utm\\_content=bufferfo626&utm\\_me](http://www.pcori.org/events/2016/health-affairs-briefing-patients-and-consumers-use-evidence?utm_content=bufferfo626&utm_me)

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### **Patient-Reported Outcomes version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE™) is now available for use in clinical trials**

The National Institutes of Health (NIH) National Cancer Institute (NCI) – Division of Cancer Control & Population Sciences has developed the measure to evaluate symptomatic toxicity in patients on cancer clinical trials.

The PRO-CTCAE was designed to be used in clinical trials as a companion to the Common Terminology Criteria for Adverse Events (CTCAE) – the standard method for clinicians to report adverse events in NCI-sponsored clinical trials – to capture symptomatic treatment side effects from the patient perspective using a systematic yet flexible approach. The PRO-CTCAE includes an item library of 124 items representing 78 symptomatic toxicities.

Visit <http://healthcaredelivery.cancer.gov/pro-ctcae> for more information about the PRO-CTCAE

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### **New publications on public and patient engagement**

We are very grateful to François-Pierre Gauvin for sharing the following Bulletin of recent publications that he prepares. The [Partnership Lab](#) (lead by Dr Antoine Boivin) produces the monthly electronic newsletter that seeks to facilitate access to new publications.

#### **April 2016**

Engagement en recherche / Engagement in research

[What is going on in public involvement in health research? A qualitative exploration of aims, processes and outcomes](#) (Libre accès/Open access)

Davies R (2016) *University of the West of England*

[Exploring the challenge of health research priority setting in partnership: Reflections on the methodology used by the James Lind Alliance Pressure Ulcer Priority Setting Partnership](#) (Libre accès/Open access)

Madden M & Morley R (2016) *Research Involvement and Engagement*

[Evaluating public involvement in research design and grant development: Using a qualitative document analysis method to analyse an award scheme for researchers](#) (Libre accès/Open access)

Baxter S et al. (2016) *Research Involvement and Engagement*

[Going the extra mile – Creating a co-operative model for supporting patient and public involvement in research](#) (Libre accès/Open access)

Horobin A (2016) *Research Involvement and Engagement*

[Prioritising target behaviours for research in diabetes: Using the nominal group technique to achieve consensus from key stakeholders](#) (Libre accès/Open access)

Mc Sharry J et al. (2016) *Research Involvement and Engagement*

[A patient and public involvement \(PPI\) toolkit for meaningful and flexible involvement in clinical trials – a work in progress](#) (Libre accès/Open access)

Bagley H et al. (2016) *Research Involvement and Engagement*

[Determining the research priorities for patients with chronic kidney disease not on dialysis](#)

Hemmelgarn BR et al. (2016) *Nephrology Dialysis Transplantation*

Positive reporting? Is there a bias in reporting of patient and public involvement and engagement?

Chew-Graham C (2016) *Health Expectations*

A critical analysis of the implementation of service user involvement in primary care research and health service development using normalization process theory

Tierney E et al. (2016) *Health Expectations*

Framework for enhancing clinical practice guidelines through continuous patient engagement

Armstrong MJ et al. (2016) *Health Expectations*

Rethinking the relationship between science and society: Has there been a shift in attitudes to Patient and Public Involvement and Public Engagement in Science in the United Kingdom?

Boaz A et al. (2016) *Health Expectations*

Involving patients in clinical research: The Telescot Patient Panel

Fairbrother P et al. (2016) *Health Expectations*

Engaging patients as partners in developing patient-reported outcome measures in cancer: A review of the literature

Camuso N et al. (2016) *Supportive Care in Cancer*

Moving beyond the rhetoric of patient input in health technology assessment deliberations

Wortley S et al. (2016) *Australian Health Review*

Engagement dans la transformation des systèmes de santé / Engagement in health-system transformation  
Health Affairs (Numéro spécial / Special issue) >> Patients' & Consumers' Use Of Evidence

Livre / Book >> Stewart E (2016) *Publics and Their Health Systems : Rethinking Participation*

Assessing the organizational impact of patient involvement: A first STEPP

Kreindler SA et al. (2016) *International Journal of Health Care Quality Assurance*

Convier les citoyens des quartiers populaires: Prendre en compte leur expertise et favoriser leur pouvoir d'agir

Laporte A et al. (2016) *Revue d'épidémiologie et de santé publique*

Health policy: Addressing conflicts of interest of public speakers at advisory committee meetings

McCoy MS & Emanuel EJ (2016) *Nature Reviews Clinical Oncology*

Patient involvement in the development of a handbook for moderate rheumatoid arthritis

Prothero L et al. (2016) *Health Expectations*

Engagement dans l'enseignement / Engagement in teaching

Medical students' experience: Community engagement and advocacy through photovoice

Hunter HC et al. (2016) *International Journal of Medical Students*

The state of community engagement in graduate education: Reflecting on 10 years of progress (Libre accès/Open access)

Morin SM et al. (2016) *Journal of Higher Education Outreach and Engagement*

### March 2016

Engagement du public et des patients en recherche

Health and medical research funding agencies' promotion of public engagement within research: A qualitative interview study exploring the United Kingdom context

van Bekkum et al., *Health Research Policy and Systems*, 2016

Mobilisation for public engagement: Benchmarking the practices of research institutes.  
Entradas & Bauer, *Public Understanding of Science*, 2016

Engaging older adults in healthcare research and planning: A realist synthesis  
McNeil et al., *Research Involvement and Engagement*, 2016

Three conceptual models of patient and public involvement in standard-setting: From abstract principles to complex practice  
Knaapen & Lehoux, *Science as Culture*, 2016

Community involvement in development of evidence-informed recommendations for rehabilitation for older adults living with HIV  
Solomon et al., *Progress in Community Health Partnerships: Research, Education, and Action*, 2016

Monitoring and evaluation of patient involvement in clinical practice guideline development: Lessons from the Multidisciplinary Guideline for Employment and Severe Mental Illness, the Netherlands  
van der Ham et al., *Health Expectations*, 2016

Patients as partners: Building on the experience of Outcome Measures in Rheumatology (OMERACT)  
Kirwan et al., *Arthritis & Rheumatology*, 2016

Transdisciplinary tour-de-force: The Canadian National Transplant Research Program  
Hébert et al., *Transplantation*, 2016

Involving patients in health technology funding decisions: stakeholder perspectives on processes used in Australia  
Lopes et al., *Health Expectations*, 2016

Preferences for engagement in health technology assessment decision-making: A nominal group technique with members of the public  
Wortley et al., *BMJ Open*, 2016

The language of engagement: "Aha!" moments from engaging patients and community partners in two pilot projects of the Patient-Centered Outcomes Research Institute...  
Tai-Seale et al., *The Permanente Journal*, 2016

A SHARED study-the benefits and costs of setting up a health research study involving lay co-researchers and how we overcame the challenges  
Mockford et al., *Research Involvement and Engagement*, 2016

Using complexity principles to understand the nature of relations for creating a culture of publically engaged research within Higher Education Institutes  
Durie et al., *Open Research Exeter*, 2016

Combining PPI with qualitative research to engage 'harder-to-reach' populations: Service user groups as co-applicants on a platform study for a trial  
Morgan et al., *Research Involvement and Engagement*, 2016

It's not evidence, it's insight: Bringing patients' perspectives into health technology appraisal at NICE  
Staley & Doherty, *Research Involvement and Engagement*, 2016

Opportunities, challenges and ethical issues associated with conducting community-based participatory research in a hospital setting  
Strike et al., *Research Ethics*, 2016

Continuous patient engagement in cardiovascular disease comparative clinical effectiveness research.  
Vandigo et al., *Expert Review of Pharmacoeconomics and Outcomes Research*, 2016

Patient and caregiver priorities for outcomes in hemodialysis: An international nominal group technique study  
Urquhart-Secord et al., *American Journal of Kidney Disease*, 2016

Patient-reported quality indicators for osteoarthritis: A patient and public generated self-report measure for primary care  
Blackburn et al., *Research Involvement and Engagement*, 2016

Dissemination as dialogue: Building trust and sharing research findings through community engagement  
McDavitt, *Preventing Chronic Disease*, 2016

Engaging the public with your research  
Paul & Motskin, *Trends in Immunology*, 2016

What difference does patient and public involvement make and what are its pathways to impact? Qualitative study of patients and researchers from a cohort of randomised clinical trials  
Dudley et al., *PLoS One*, 2015

Engagement du public et des patients dans l'élaboration de politiques et l'organisation des services  
International Review of Administrative Sciences >> Special issue: Coproduction of public services  
o Co-production in healthcare: Rhetoric and practice  
Vennik et al., *International Review of Administrative Sciences*, 2016

Revue Internationale des Sciences Administratives >> Numéro spécial sur la coproduction des services publics  
o La coproduction dans les soins de santé : le discours et la pratique  
Vennik et al., *Revue Internationale des Sciences Administratives*, 2016

Patient involvement in quality improvement  
Gustavsson, *Doctoral thesis* -, 2016

NHC's patient-centered value model rubric  
National Health Council, 2016

Service user and caregiver involvement in mental health system strengthening in low-and middle-income countries: Systematic review  
Semrau et al., *BMC Health Services Research*, 2016

Conceptions of patients and their roles in healthcare: Insights from everyday practice and service improvement  
McDermott & Pedersen, *Journal of Health Organization and Management*, 2016

The role of patient narratives in healthcare innovation: Supporting translation and meaning making  
Pedersen, *Journal of Health Organization and Management*, 2016

What patients do and their impact on implementation: An ethnographic study of participatory quality improvement projects in English acute hospitals  
Boaz et al., *Journal of Health Organization and Management*, 2016

Les usagers résistent en prenant la parole  
Deutsch - *VST-Vie sociale et traitements*, 2016

Are we really patient focused? Time to challenge ourselves  
Drenkard, *Journal of Nursing Administration*, 2016

Service user involvement in mental health care: An evolutionary concept analysis  
Millar et al., *Health Expectations*, 2016



From tokenism to empowerment: Progressing patient and public involvement in healthcare improvement  
Ocloo & Matthews, *BMJ Quality & Safety*, 2016

Patient participation in contemporary health care: Promoting a versatile patient role  
Souliotis, *Health Expectations*, 2016

Engagement du public et des patients dans l'enseignement  
When patients train doctors: Feasibility and acceptability of patient partnership to improve primary care providers' awareness of communication barriers in family medicine for persons with serious mental illness  
Pelletier et al., *Mental Health and Family Medicine*, 2016

The patient's voice in health and social care professional education: the Vancouver Statement  
Towle et al., *International Journal of Health Governance*, 2016

Where's the patient's voice in health professional education?  
Towle, *Interface-Comunicação, Saúde, Educação*, 2016

Opening the black box: Conceptualizing community engagement from 109 community-academic partnership programs  
Ahmed et al., *Progress in Community Health Partnerships: Research, Education, and Action*, 2016

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Janet Wale, Chair HTAi PCIG  
E-mail: [socrates111@bigpond.com](mailto:socrates111@bigpond.com)